Gene Therapy

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Deffination

Is one of the applications of genetic engineering may contribute to healing of many diseases of genetic as Hemophilia and autoimmune diseases as rheumatoid arthritis, cystic fibrosis and chronic diseases such as cancer and infectious diseases such as AIDS. Or is the treatment of a disease by replacing the gene to be another sound Gene replacement

Or supply of the patient's cells sufficient number of healthy gene transfer of these genes are required to work and to compensate the patient for the shortfall in the work of the damaged genes.

Or is to replace defective genes with another sound to treat the disease or the introduction of healthy genes carrying genetic information into unwanted cell gene is therefore in this case as medicine. Gene therapy offers the possibility of providing the human body the same ability to synthesize certain substances (drugs) and also the time "with the possibility of stabilizing treatment for life. Returns the first experiment to use gene therapy to the year 1995 when the two doctors V. Anderson and Michael Blaz trying to treat a child infected with immunodeficiency common severe enter inherited competent to strengthen the immune system in the human body gained experience partial success where he was able therapy to strengthen the immune system of a child by 40%.

The gene therapy of experimental techniques that are used for treatment or prevention of disease through the insertion of genetic material and entered into the cell to compensate for abnormal genes and when the mutant gene causing the loss and alteration of a protein necessary for the functions of the natural and the gene therapy be able to insertion of a normal copy of the gene to restore function of the protein.

Uses of gene therapy in the treatment of serious diseases

There are many attempts for gene therapy outside the body for diseases that affect blood cells, such as thalassemia and sickle cell anemia and leukemia and hemophilia and employ a method of gene therapy outside the body for diseases other than diseases of the blood are used to treat metabolic diseases inborn error of metabolism, which often that result from the lack of systems and specific functional body as a result of defect in the gene for this system are also "in this way to add healthy gene laptop on the conveyor into the cells of the bone outside the body and then returned to the patient

has been done to address the disease, the accumulation of Alphenanal Keaton urinary (phenyl ketonuriai) . The gene therapy within the body, it is for diseases that are difficult to obtain cells or that are not divided as much or which have no stem cells, stem cell, where is the delivery of the healthy gene or laptop directly to the tissue affected, for example, treatment of fibrosis, lung fibrosis lung cystic fibrosis and patients with disorders muscle is used gene therapy in the treatment of patients with cancer, HIV and peripheral vascular disease and treatment of diabetes or diabetes, rheumatoid arthritis, and arterial stenosis, and some neurological diseases such as Alzheimer's and Parkinson's syndrome and others.

The principle of gene therapy and types of

The idea of gene therapy to introduce inherited effective and functionally to the cells by loading the legacies to an intermediary known as the carrier that the carrier after the enumeration of target cells and the introduction of the gene to the cell, thereby restoring the production of protein lost can enumerate the target cells either by extracting the cells and transplantation of a population outside the neighborhood and then re-implanted into the patient's body or directly within the neighborhood.

Has been used many ways in gene therapy, including replacement of the gene and cause a pathological condition copies of normal genes or revoked and spoilage gene mutant that does not work correctly as well as introduce new genes into the body to help in the battle against the disease and there are now more than (900) Protocol or a method of gene therapy was conducted on more than (6000) patients underwent experimental tests so far.

Despite some successful experiences of others but gene therapy is still considered a dream that flirts with the world for the treatment of many diseases, therefore, that the number of companies interested in research in gene therapy is increasing around the world. The back of the first product for the treatment of cancer gene to light in China in (2004) under the name (Gndicine), a gene (P53), which inhibit tumor formation laptop on the bus virus rate when injecting this medicine tumor the virus to enter the gene into the cancer cells, which urges the cell to kill itself.

Interestingly, it has been tested Gendsin situations late in squamous cell head and neck, and after continued treatment for a period of (8) weeks by one injection per week results showed that more than (164) of patients were completely cured, and (32%) showed the decline in part to the disease and if Gendsin use with chemotherapy or radiotherapy treatment, the efficiency increases to three-fold.

Can determine the basics of gene therapy as follows:

1 - identify the damaged gene on the site and which is intended to compensate for transfer gene addition or substitution.

2 - Gene replacement need to provide the healthy gene to be give to the patient this has been available for half the number of human genes thanks to scientific advances in the techniques of recombination of DNA Technology recombinant DNA are the genes carried on vectors and cloned after the completion of the Human Genome Project has become affordable for any gene required.

3 - provide a mechanism for gene delivery to target cells in addition to access to the target cells.

4 - need not be cause for a genetic mutation a new result to enter the gene given results in disabling the gene active or activate the forefront of the oncogene proto-oncogene into Jenna and shot, or cause to disrupt the gene inhibitor of tumor suppressor gene to unleash the oncogene and the damage the latter are more likely than The first and possibly damage the latter is the possibility that the working gene is given in the cells of other non-target cells, which is caused by the bad consequences if the gene Beta globin who transferred to marrow cells Thalassemia (B-thalassemia) in the white blood cells in the time that must hold only in the red blood cells.

5 - result in an improvement in the patient's condition and up the healthy gene into a number of target cells and remain in the Expressed and is expressed to be given any result.

Of the most important problems that hinder the success of gene therapy is a sample delivery of the healthy gene to the target cells and reach sufficient numbers and to a sufficient number of diseased cells, as well as a new gene in the case of the stability and do not break as well as be able to express himself which produces a protein, to achieve anything that has to be and a holder of these genes can achieve the stated objectives, which has the natural property to enter the cells is virus .

Types of gene therapy

Divides the gene therapy based on the target cells into two parts:

1 - Gene therapy of somatic cells (Somatic Gene therapy)

Any repair any genetic defect at the level of all the cells of the body of the person just the patient, except the sex cells (sperm in the male and the egg in the female) as well as the fertilized egg ((Zygote is gene therapy through the use of treatment somatic cell Dealing with genetic changes in somatic cells of human to treat certain disorders, as is extracted cells infected person and processed outside the body in

vitro therapy, or in some cases, treatment of cells is in the body in vivo with the observation that some types of somatic cells to be obedient for gene therapy than others It is preferable that the cells are candidates for gene therapy distinct period of long life in the body with easy access to it.

2 - Gene Therapy at the level of sex cells (Germline Gene therapy) Where the treatment of the female egg or sperm of a male or a fertilized egg (zygote) in the first stages of growth, and before that differentiate into specialized cells.

Both methods differ in the consequences are incurred after treatment of the cells genetic sexual or zygote always results in a change in the genotype of the patient to the processor Agency.

As well as scientific sources indicated that he could be gene therapy to replace the gene (Gene Replacement therapy) , where after one of the most widely used techniques in gene therapy. It is replaced by the defective gene that causes the loss or alteration of a protein necessary a copy of the gene is normal and insert it into the somatic cells and the fact that many disorders and illnesses produced can be corrected in a large production of small amounts only of the gene, the gene therapy, even if its effect in part may give 5% to 20% of the amount of production of the natural gene, it will be enough to get significant health benefits and moral.

As the sources of scientific, there several techniques to introduce the gene into the cell, including the integration and fusion of the cell fusion and the treatment calcium phosphate as the cause calcium phosphate interference and disruption in the cell membrane, allowing for the DNA of a negative charge to overcome the repulsion natural chemical to the cell membrane negative charge and soundly-mail Electro parathion

This is done by exposing the cell electric shock, which allows the passage of DNA through the strange and the cell membrane fusion or docking of the body lipid liposome fusion and direct entry of DNA naked DNA Kalona after cloning a piece of human DNA in the plasmid.

How is gene therapy

Is by:

1 - stop the work of the damaged gene

And it will be off work the gene damaged and prevent the secretion of the protein that causes the disease is through the movement at any stage of the expression of the gene and prevent the secretion of the protein that causes the disease by introducing pieces of DNA do not carry any genetic information to combine with gene damaged and

prevent the production of protein.
2 - Insert a copy of the gene intact
And it is a sound introduction of the gene to be expressed and which, in turn, patients will be treated even in the case of a damaged version of the gene, there are two ways to enter: -
A - out of the body : and which is taking the cells you want to enter the gene in which the body then is inserted gene is desired in the cells with culturing these cells in the special circumstances and then be re-cells adjusted again to the body Examples of such cells, muscle cells tumor cells - bone marrow - white blood cells, liver cells.
B - within the body : and it is entered version sound of the gene directly to the cell in the human body using the delivery systems of certain without having to remove those cells outside the human body and therefore this method is suitable for cells difficult to obtain, such as brain, lung and heart is Virus best carriers vital Biological vector and the type used is to viruses regressive Retro viruses which of these viruses from the property access to the cells of the body and the insertion in the chromosomes of the human becomes part of the genes of the person the processor by which the genetic material of these viruses is RNA rather than DNA and when you enter these viruses to the cells become DNA and RNA to DNA implanted in the person and the future become part of the natural genetic composition.
Vectors are of two types:
1 – Viral Vectors
2 – Non Viral Vectors

1 - viral vector

Most of the viruses carrying the genetic content of the cell with the target as part of their life cycle and the scientists used genetically engineered viruses as a means of gene transfer to the target cells of viruses that have been used as vectors :
Viruses, adenoviruses and the viruses associated with viruses adenoviruses and the viruses, retroviruses, there are some problems occur when you use redundancy viral, including restoration of virus activity morbidity him and thus injury ill again, as happens in a clinical trial when the injury (3), (9) patients with disease, leukemia and problems also there were "no guarantees that the redundancy is used to target the desired cells or viruses enter the cell's genetic material in the place of others is true and therefore render useless the work of other heritage.
Types of viruses that carry the genetic material RNA is a Virus reverse (retroviruses) of viruses that carry the genetic material RNA, where the trapped and integrate its genetic material, including the

gene that are moving with the tape double DNA chromosomes of a human cell and must adapt and adjust Virus reverse using the techniques of re-installation of the genetic material (Recombinant DNA) techniques.
To remove or delete most of the genome, the virus and replaced version of natural human gene to be used in gene therapy with the regulatory elements of this gene as well as for signs of urinary Dinellas (poly denylationsignal) called the genetic material in Annex insert viruses inverse able to reduce the supplements size up to 8,000 base nitrogenous kb8 Virus is then reverse-lap modified with the somatic cells of the patient.
Such as stem cells for bone marrow Bone marrow stem cell and lymphoid cells lymphocytes are modified viruses to plant gene in normal human DNA of these cells.

2 - non viral vectors

Used to transfer genes to the cell, including objects fat or that can reduce the supplements a large volume of DNA or happen merger or fusion of body fat with the cell, allowing for the supplements to the DNA to enter the cell and does not contain objects lipid proteins, it does not stimulate the body's immune response, thereby hindering the use of body fat in gene therapy that most of these objects are analyzed in the cell cytoplasm and degrade even can not enter the nucleus.

The Warning of the gene therapy

Issues of medical, social and ethical about gene therapy holds the most important scientists in the light of the foregoing is that the genetic effects of this treatment is expected, including:
1 - to plant the new gene in the wrong place or in the gene sequence and cause the sound off or disabled to work.
2 - to plant gene mobile gene inhibitor of cancer tumor suppressor and stop him from work and so off the cells from it's stability and grow the growth of cancerous or cause the planting wrong in activating the forefront of gene tumor on which the non-active state proto-oncogene and converts it to Jane bulging oncogene addition access the transferred gene to the cells that cause reproductive changes is based, resulting in low moral and social implications of unexpected
Of the most important problems facing society, because in the use of gene therapy at the level of germ cells is likely to result from this therapy genetic change of the cell's reproductive sperm or egg and then transfer that change to future generations and thus genetic change will mean changing the genotype of the human being forever

with the assumption It is best, but any line not in the account will be dire consequences and therefore must be handled very carefully with gene therapy.

The diseases can be treated with gene therapy techniques

1 - Cystic fibrosis disease

Of the most pathogenic genetic lethal common in whites and arises as a result of a defect in the gene responsible for manufacturing the enzyme, which causes deficiency to the formation of a thick layer of mucus lining of the digestive system and the airways leading to an imbalance in the secretion of digestive enzymes in the digestive system and a defect in the absorption of food by the channel tract as well as cause deadly respiratory infections and can cure the disease gene through the introduction of sound copy of the gene responsible for the secretion of this enzyme by inhalation.

2 - HIV genetic (SCIDs)

Syndrome Severe Combined immunodeficiency.

Disease, a fatal hereditary disease that happens as a result of a defect in the gene responsible for the secretion of the enzyme Adenosine de-aminase enzyme

Responsible for some immune functions can be treated this disease by introducing a healthy copy of the gene into white blood cells.

3 - Cancer

Gene can be treated either by stopping the work of the gene that causes cancer or the introduction of a copy of the sound of the tumor inhibitor gene or genes using a technique in which the urging suicide cell gene on the formation of a tumor causing the destruction of the cell itself.

4 - AIDS (HIV)

Is inserted gene in cells infected with virus likely causes AIDS by cell consists substance that prevents the virus from multiplying and survival, as well as the technique can be applied to gene therapy in the treatment of many diseases such as Parkinson's disease and increased cholesterol in the blood genetic.